
Stem Cell Agency Invests in Chemotherapy-Free Approach to Rare But Deadly Childhood Disease

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Oakland, CA – Imagine being told that your seemingly healthy newborn baby has a life-threatening disease. In a moment your whole world is turned upside down. That's the reality for families with a child diagnosed with severe combined immunodeficiency (SCID). Children with SCID lack a functioning immune system so even a simple cold can prove fatal. Today the governing Board of the California Institute for Regenerative Medicine (CIRM) awarded \$3.7 million to develop a new approach that could help these children.

The funding will enable Stanford's Dr. Judith Shizuru to complete an earlier CIRM-funded Phase 1 clinical trial using a chemotherapy-free transplant procedure for SCID.

The goal of the project is to replace SCID patients' dysfunctional immune cells with healthy ones using a safer form of bone marrow transplant (BMT). Current BMT procedures use toxic chemotherapy to make space in the bone marrow for the healthy transplanted stem cells to take root and multiply. The Stanford team is testing a safe, non-toxic monoclonal antibody that targets and removes the defective blood forming stem cells in order to promote the engraftment of the transplanted stem cells in the patient.

The funding is contingent on Dr. Shizuru raising \$1.7 million in co-funding by May 1 of this year.

"This research highlights two of the things CIRM was created to do," says Maria T. Millan, MD, President & CEO of CIRM. "We fund projects affecting small numbers of patients, something many organizations or companies aren't willing to do, and we follow those projects from the bench to the bedside, supporting them every step along the way."

Early testing has shown promise in helping patients and it's hoped that if this approach is successful in children with SCID it may also open up similar BMT therapies for patients with other auto-immune diseases such as multiple sclerosis, lupus or diabetes.

About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With \$3 billion in funding and approximately 300 active stem cell programs in our portfolio, CIRM is the world's largest institution dedicated to helping people by bringing the future of cellular medicine closer to reality.

For more information go to www.cirm.ca.gov

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